## Human Satellite Cell Transplantation and Regeneration from Diverse Skeletal Muscles.

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**Funding Grants:** Characterization of Human Skeletal Muscle Stem Cells for Clinical Application

**Public Summary:**
This paper focused on identification of appropriate donor muscles for human muscle stem cell applications and characterization of endogenous human muscle stem cells. As human muscle stem cells had not been definitively characterized or transplanted, the initial main focus of this CIRM grant was to do so with stem cells from diverse muscles and, combined with our clinical understanding of expendability of different muscles, choose human muscles that will be appropriate to harvest stem cells from in future applications. We have accomplished that and published the essential results in this report.

**Scientific Abstract:**
Identification of human satellite cells that fulfill muscle stem cell criteria is an unmet need in regenerative medicine. This hurdle limits understanding how closely muscle stem cell properties are conserved among mice and humans and hampers translational efforts in muscle regeneration. Here, we report that PAX7 satellite cells exist at a consistent frequency of 2-4 cells/mm of fiber in muscles of the human trunk, limbs, and head. Xenotransplantation into mice of 50-70 fiber-associated, or 1,000-5,000 FACS-enriched CD56(+)/CD29(+) human satellite cells led to stable engraftment and formation of human-derived myofibers. Human cells with characteristic PAX7, CD56, and CD29 expression patterns populated the satellite cell niche beneath the basal lamina on the periphery of regenerated fibers. After additional injury, transplanted satellite cells robustly regenerated to form hundreds of human-derived fibers. Together, these findings conclusively delineate a source of bona-fide endogenous human muscle stem cells that will aid development of clinical applications.